PTC Therapeutics

October 2026



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Forward Looking Statements

This presentation contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this presentation, other than statements of historic fact, are forward-looking statements, including with respect to 2026 total product revenue guidance and total revenue guidance and 2026 operating expense guidance, and statements regarding: the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses, meetings with regulatory agencies, commercialization and other matters with respect to its products and product candidates; PTC's strategy, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words, "guidance," "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," "aim," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; expectations with respect to Sephience, including commercialization and the potential achievement of sales milestones and contingent payments that PTC may be obligated to make; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in geographies in which it has been approved and the effect of the European Commission's adoption of the negative opinion from the Committee for Medicinal Products for Human Use (CHMP) on Translarna and the withdrawal of the Translarna NDA in the US on other regulatory bodies; expectations with respect to PTC's license and collaboration agreement with Novartis Pharmaceuticals Corporation for votoplam for the treatment of Huntington's disease including its right to receive development, regulatory and sales milestones, profit sharing and royalty payments from Novartis, the design and expected timing of clinical trials and studies, the availability of data, and regulatory submissions and responses, including potential accelerated approval; expectations with respect to ST-920, including the expected timing of regulatory submissions and potential approvals, and the potential achievement of regulatory milestones and contingent payments PTC may be obligated to make; expectations with respect to Upstaza/Kebilidi, including commercialization, manufacturing capabilities, and the potential achievement of sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to vatiquinone, including with respect to the design and expected timing of clinical trials and studies, the availability of data, and regulatory submissions and responses and potential approvals and other matters; expectations with respect to the commercialization of Evrysdi under PTC's SMA collaboration; expectations with respect to the commercialization of Tegsedi and Waylivra; expectations regarding PTC's product candidates, including the timing of clinical trials and studies; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of PTC's products and product candidates; PTC's scientific approach and general development progress; PTC's ability to satisfy its obligations under the terms of its lease agreements; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's Annual Report on Form 10-K, as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Sephience, Translarna, Emflaza, Upstaza, Kebilidi, Evrysdi, Tegsedi or Waylivra.

The forward-looking statements contained herein represent PTC's views only as of the date of this presentation and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this presentation except as required by law.

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PTC: A Global Leader in Rare Disease Drug Development and Commercialization

Robust global commercial infrastructure

Proven track record of achieving regulatory authorization, gaining market access and commercializing rare disease therapies in over 50 countries.

Lead product Sephience supports near- and mid-term growth

Strong initial global launch and positioned to be new standard of care for PKU. IP into 2039 and $2B+ global revenue potential.

Innovative R&D platforms and pipeline

Pioneered field of oral small molecule RNA splicing with proprietary platform engine to accelerate and facilitate next generation of oral small molecule splicing therapies.

Strong financial position

Cash balance of $2.1B[1] and on track to achieve 2026 cash flow breakeven and future sustainable profitability.

[1]As of September 30, 2026

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2026 Strategic Priorities

- Continue Sephience global launch momentum
- Advance Phase 3 votoplam HD program
- Advance early-stage R&D programs
- Move toward reaching cash flow breakeven

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2026 Revenue and OpEx Guidance

Product Revenue[1] $960 - 990M

64 - 69% YoY Growth

Total Revenue[2] $1.29 - 1.32B

OpEx[3] $680 - 720M

Our Products[4]

Sephience™ sepiapterin

translarna™ ataluren

Emflaza® (deflazacort) 6 mg | 18 mg | 30 mg | 36 mg tablets 22.75 mg/mL oral suspension

Kebilidi™ (eladocagene exuparvovec-tneq)

Upstaza™ (eladocagene exuparvovec)

waylivra® (volanesorsen) injection 285 mg/1.5 mL

Tegsedi® (inotersen) injection 284 mg/1.5 mL

1 Product revenue excludes Evrysdi royalty and collaboration revenues; YoY based on $960-990M guidance.
2 Total revenue includes Evrysdi royalty and collaboration revenues.
3 OpEx is a non-GAAP measure that includes only R&D and SG&A expenses for full year 2026, and excludes estimated non-cash, stock-based compensation expense of approximately $95M. GAAP R&D and SG&A expense for full year 2026 is anticipated to be between $775 and $815M.
4 PTC holds the commercialization rights for Tegsedi and Waylivra in Latin America and the Caribbean from Ionis Pharmaceuticals.

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Sephience™
PKU Program



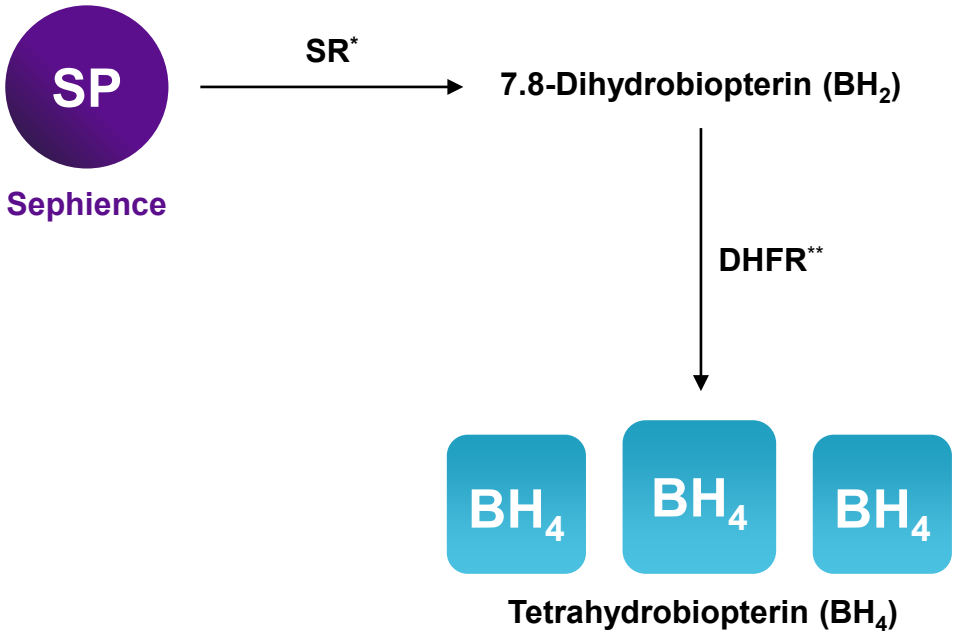
Katie, living with PKU

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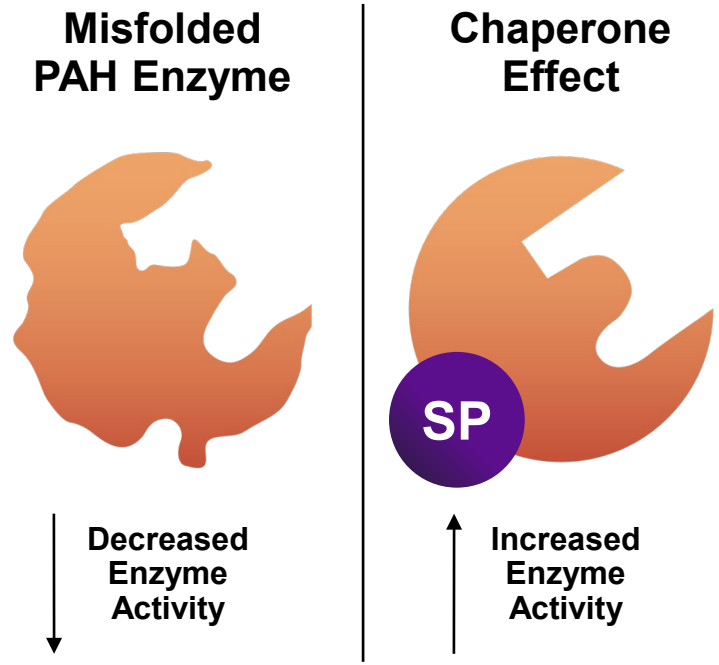
Sephience Dual Mechanism of Action Supports Potential for Broad Uptake

1. Precursor to BH_4



Sephience significantly increases intracellular BH_4 levels

2. Independent Chaperone Function

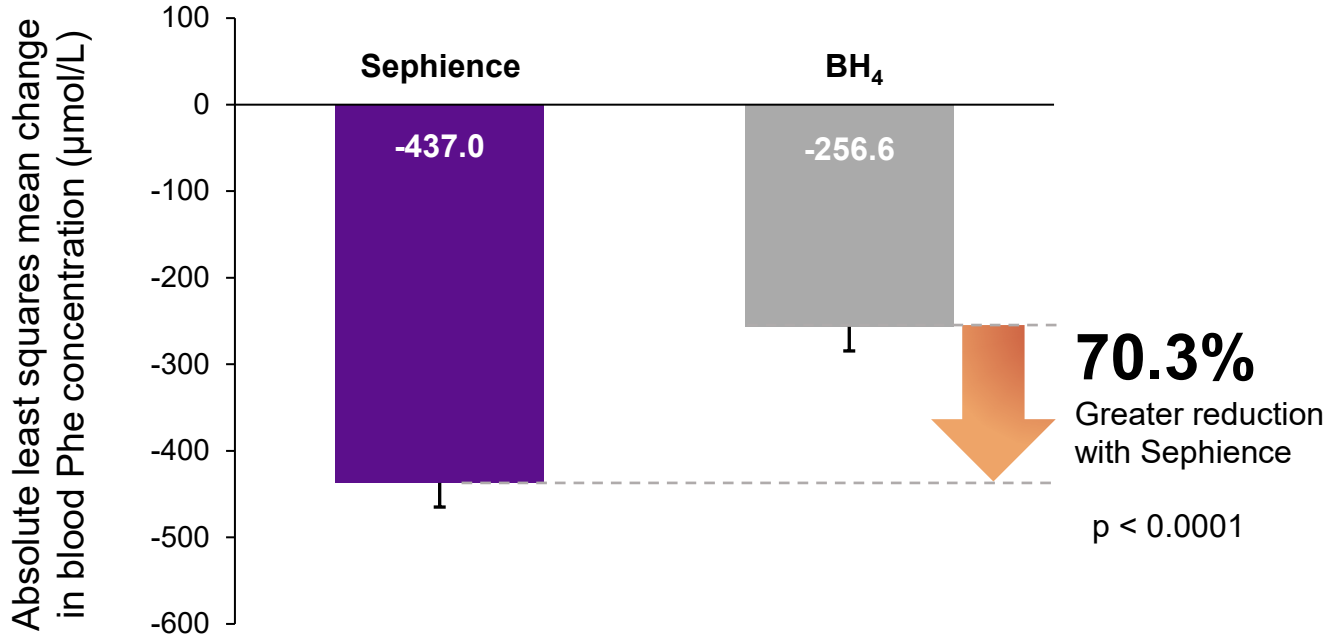


Chaperone effect stabilizes enzyme, increases function, and enables treatment of BH_4-non-responsive mutations

* SR, sepiapterin reductase (enzyme that converts Sephience to BH_2)
** DHFR, dihydrofolate reductase (enzyme that converts BH_2 to BH_4)
Lah, M. Advances in Therapy (In press)

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Sephience Provides Significantly Greater Phe Reduction Compared With BH_4



AMPLIPHY was a crossover study allowing for intra-subject comparison of Sephience and BH_4 treatment effect

Lancet. 2024 Oct 5;404(10460):1333-1345. doi: 10.1016/S0140-6736(24)01556-3.

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Sephience Provides Diet Liberalization, Cognitive and Quality of Life Benefits

aphenity extension study

Meaningful diet liberalization

69% of subjects reached age-adjusted protein RDA

Improved cognitive function and mood

Improved concentration & slow thinking, and decreased irritability

Improved quality of life

Reduced disease impact on emotional, social, and familial wellbeing

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Genet Med. 2026 Apr;28(4):101683. doi: 10.1016/j.gim.2026.101683. Epub 2026 Jan 12.

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Sephience Differentiated Profile Enables Penetration Into All Key Market Segments



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Sephience: Strong Launch Momentum Continues



Broad penetration into all key market segments

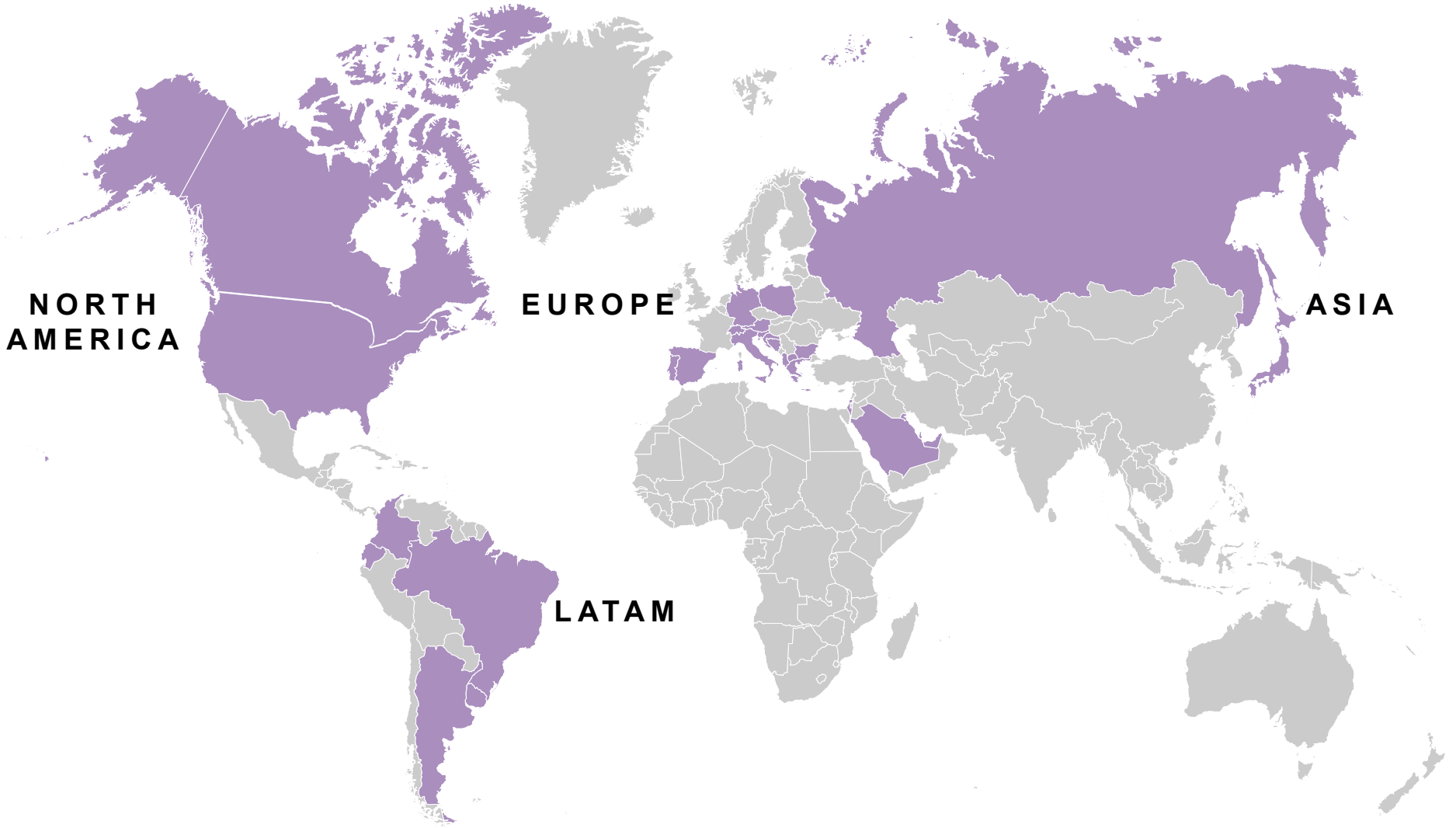
$458M Global revenue YTD in first full year of launch

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* As of September 30, 2026

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Sephience Global Launch Footprint Expansion Planned for 2026 and 2027



Sephience™ sepiapterin

Anticipated Sephience geographic footprint for 2026

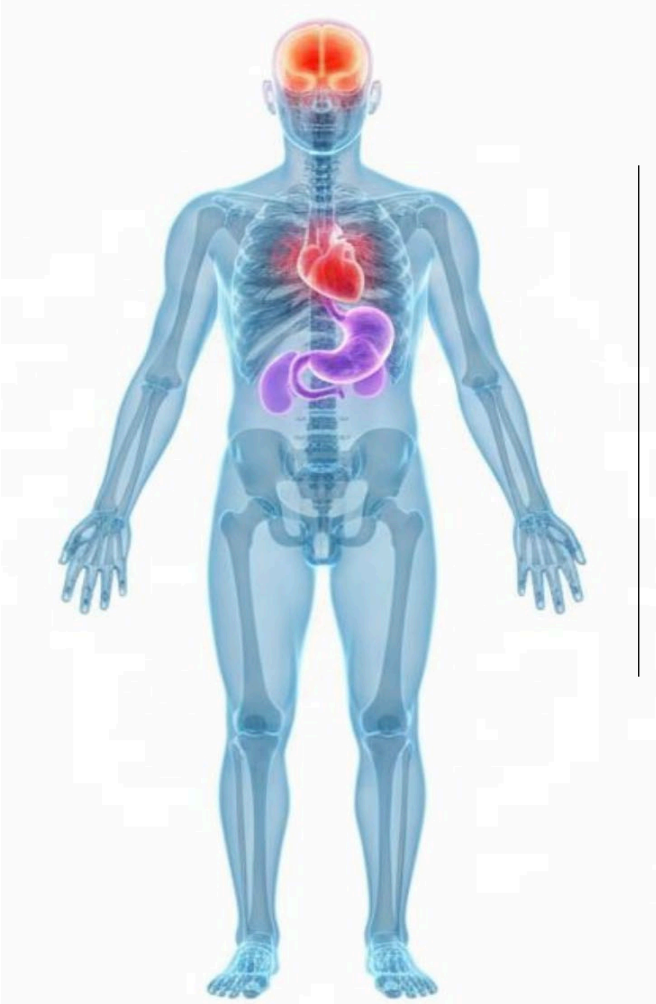
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ST-920
Fabry Disease Program

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Fabry Disease is a Rare, Life-Shortening Disorder with Multiorgan Impact

- Lysosomal storage disorder causing serious organ damage, leading to premature death
- Affects ~11,000 individuals (US)
- Standard of care of enzyme replacement therapies (ERT) have several drawbacks
  - Frequent (up to biweekly) administration
  - Require immunosuppression
  - Risk of antibody formation limiting ERT benefits
- A one-time administered gene therapy has potential to provide benefits of ERT without current burdens and risks



Organ involvement includes kidney, heart and brain

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ST-920[1] Provides One-Time Administered Durable Enzyme Production

- AAV gene therapy that provides humanized gene enabling long-term production of the deficient α-Gal A enzyme
- Favorable safety profile with no need for immunosuppression
- Durable α-Gal A activity with longest follow up of over 5 years
- Favorable effects on renal function and other disease aspects following gene therapy administration

BLA submission for accelerated approval expected to be completed in Q4 2026

1 Isaralgagene civaparvovec

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Votoplam

Huntington’s Disease Program



April, living with HD

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Phase 2 PIVOT-HD Trial Achieved Key Efficacy and Safety Objectives

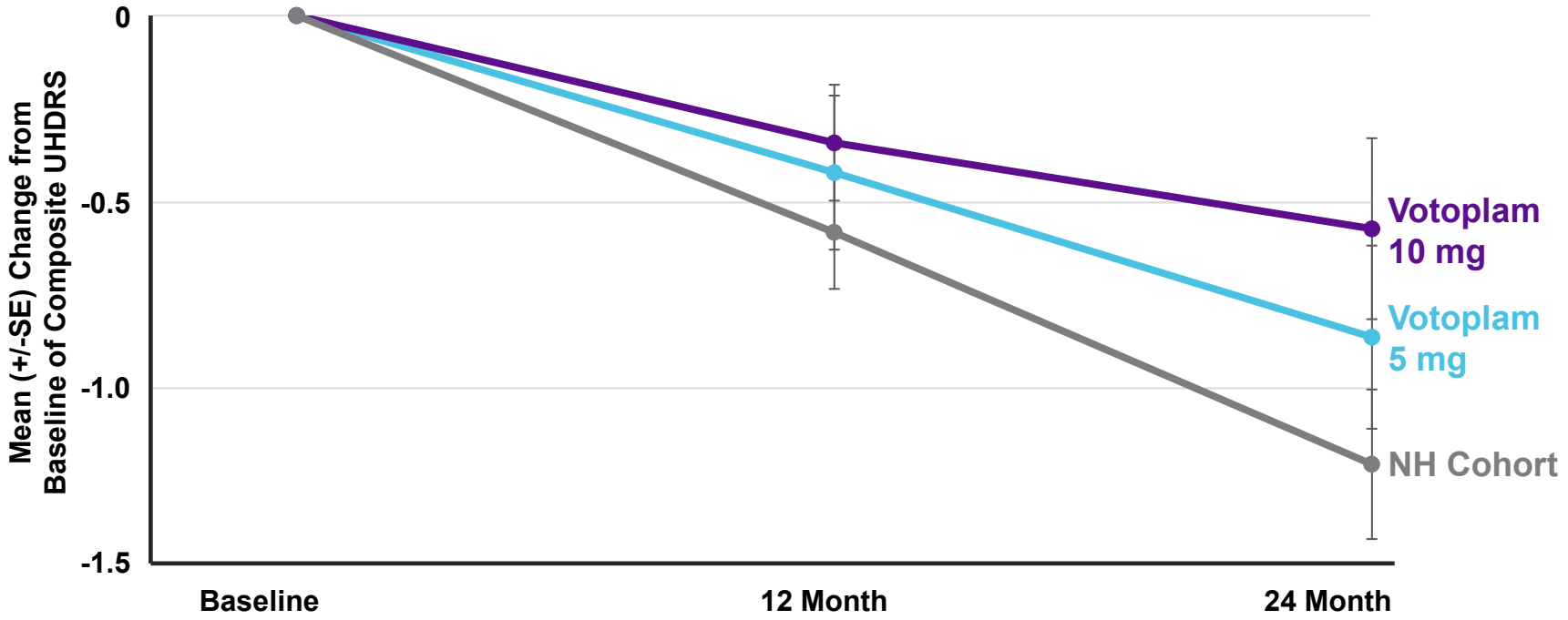
PIVOT-HD study met primary endpoint of blood HTT protein lowering at Week 12 with durable dose-dependent lowering at Month 12

Evidence of dose-dependent disease slowing on cUHDRS in Stage 2 participants at 24 months

Continued evidence of favorable safety profile with no new AE signals identified for both dose levels and stages at 24 months

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Dose-dependent Slowing of Disease Progression on cUHDRS at 24 months in Stage 2 Participants



Treatment Group	Mean (SE)	Mean (SE)
Natural History (N=73)	-0.58 (0.15)	-1.20 (0.20)
5 mg (N=21)	-0.42 (0.16)	-0.86 (0.24)
10 mg (N=24)	-0.34 (0.21)	-0.57 (0.24)

Results based on observed data
SE = Standard Error

Mean difference in cUHDRS progression of 52% (10mg) and 28% (5mg) at Month 24

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INVEST-HD Global Phase 3 Trial Enrolling Worldwide

Study Overview

- Individuals with early symptomatic disease
- 3:2 randomization of votoplam 10mg: placebo
- Target enrollment: ~770 participants in >30 countries
- Primary endpoint: Change from baseline in cUHDRS
- Treatment period up to 36 months
- Interim analysis planned for efficacy and futility

*Study sponsored and funded by Novartis; NCT#: NCT07326709

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Vatiquinone

Friedreich’s Ataxia Program



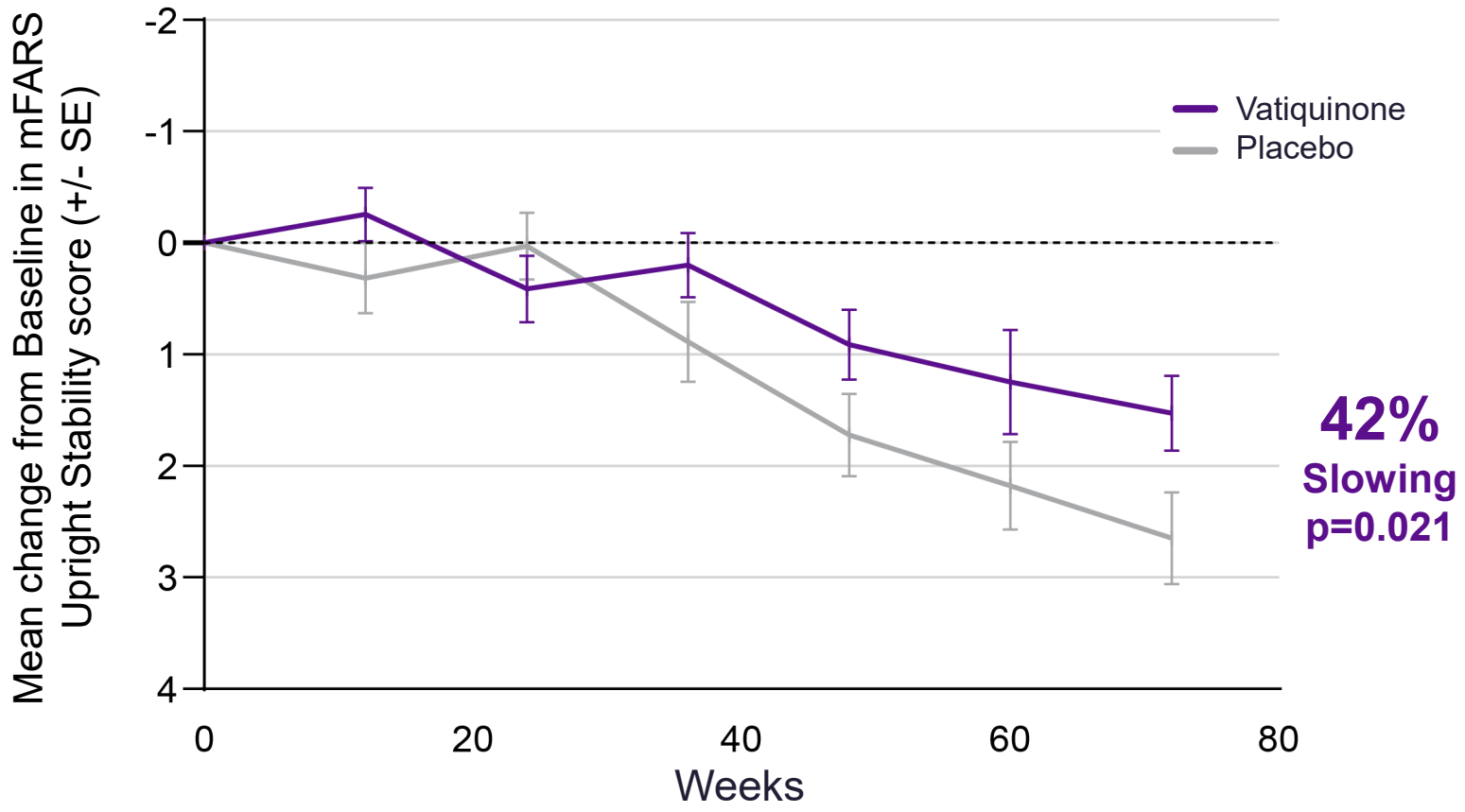
Olivia, living with FA

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Vatiquinone Demonstrated Meaningful Slowing of Short- and Long-term Disease Progression

72-week Placebo-Controlled MOVE-FA Trial*



Long-term Extension Studies

50% slowing (p<0.0001) of disease progression over 3 years** in MOVE-FA long-term extension study

4.8-point benefit (p<0.0001) on mFARS** over 2 years in ambulatory and non-ambulatory adults

* MOVE-FA did not meet its primary endpoint of statistically significant change in total mFARS at 72 weeks
** Relative to matched FACOMS (Friedreich's Ataxia Clinical Outcome Measures Study) natural history cohort

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PROVE-FA Phase 3 Open-Label Study to Support NDA Resubmission Initiated in Q3 2026

Study Overview

- Open-label study using matched natural history cohort from FACOMS disease registry
- Target enrollment: 120 individuals with FA ages 7 to 21
- Primary endpoint: Change in mFARS from baseline to 24 months



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Innovative Research Platforms



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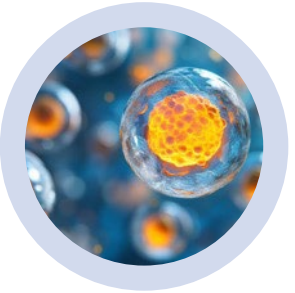
R&D Pipeline Includes Innovative Programs from Differentiated Scientific Platforms



Oral Small Molecule Splicing Platform

- **MSH3:** Huntington's Disease, Myotonic Dystrophy I
- **Undisclosed:** Brain Tumors & Metastases
- **Undisclosed:** Sickle Cell Disease, β-Thalassemia
- **Undisclosed:** Alzheimer's Disease
- **Undisclosed:** Neurodegenerative Diseases

Inflammation/ Ferroptosis Platform



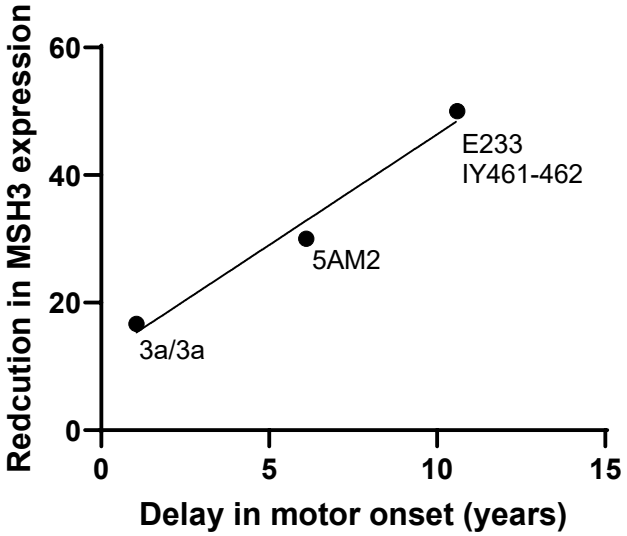
- **Ferroptosis:** Multisystem Atrophy (MSA) & Parkinson's Disease
- **NRF2 Activation:** CNS, Non-CNS Indications
- **NLRP3 Inflammasome:** Inflammatory Lung Diseases
- **DHODH Inhibition:** T-Cell Mediated Autoimmune Diseases

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PTC303 MSH3 Small Molecule Splicing Program

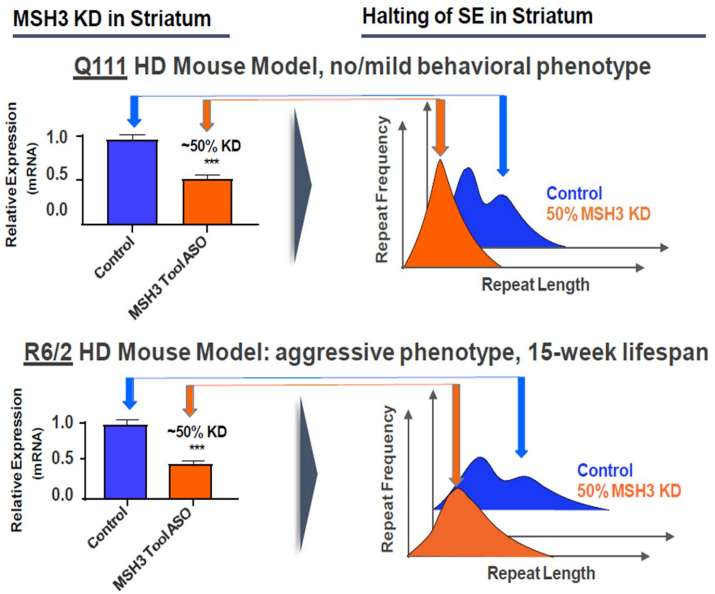
- MSH3 is a genetic modifier of repeat expansion disorders such as HD and DM1
- Validated correlation between MSH3 reduction and HD onset and progression, supporting therapeutic potential of MSH3 reduction
- PTC303 to enter clinic in 2027

Clear linear correlation between magnitude of MSH3 reduction and HD progression in patients[1]



Similar correlation for DM1

In HD mice, 50% knockdown of MSH3 halts somatic expansion in the striatum[2]



1. Flower. M et al. Brain. 2019 Jun 19;142(7):1876–1886. 2. Antonijevic, I. A. et al. (2022)

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PTC612 NLRP3 Inhibitor Program

- NLRP3 is an intracellular sensor protein that detects infection, stress or tissue damage
- Upon detection, NLRP3 is activated, triggering the assembly and activation of the NLRP3 inflammasome leading to elevated levels of IL-1β and IL-18
- Aberrant NLRP3 activation is implicated in a wide range of inflammatory diseases
- PTC612 is differentiated from other NLRP3 inhibitors in terms of potency, selectivity and chemical structure
- PTC612 Phase 1 SAD/MAD healthy volunteer study ongoing including cohort of individuals with metabolic syndrome to provide initial evidence of PK/PD effect

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PTC612: Greater Potency & Selectivity Compared to Other NLRP3 Inhibitors

PTC612 Demonstrates >10x Potency and Selectivity vs Other NLRP3 Inhibitors

Assay	PTC612	Selnoflast[1]	DFV890[2]	Usnoflast[3]	AZD4144[4]
THP-1 IL1β IC_{50} (nM)	**0.99**	220	13	13	23-82

Assay	PTC612	Vent-01[5]	DFV890[2]	VTX-3232[6]	VTX-2735[6]
hWB IL1β IC50 (nM)	**9.7**	124	180	13	80

1. In house data; 2. JMC 2025; 3. Brain Res 2024; 4. JMC 2025; 5. Inflammasome Summit 2022; 6. Ventyx Corporate Presentation June 2023

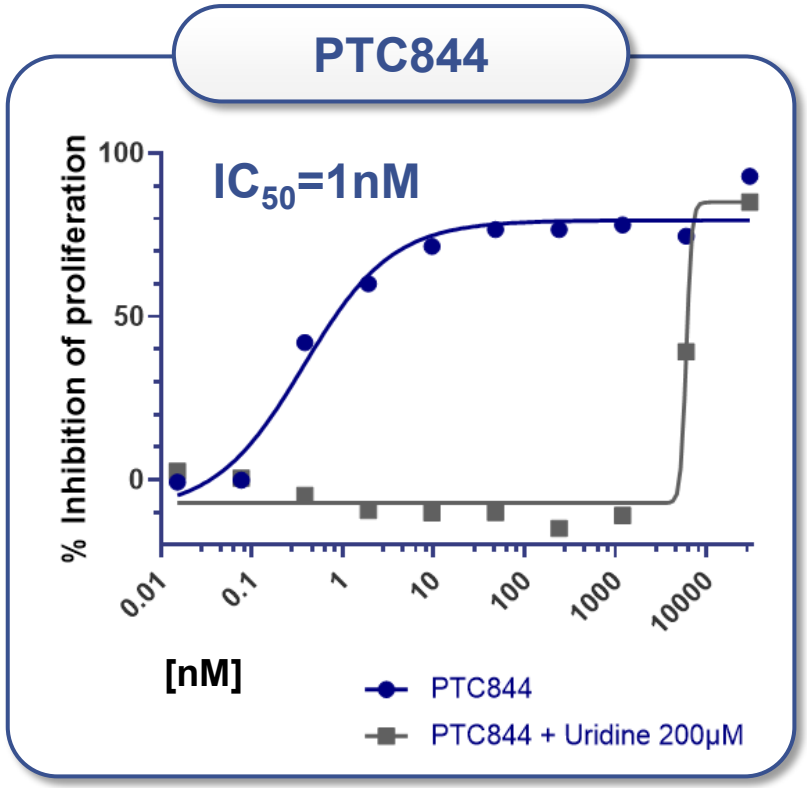
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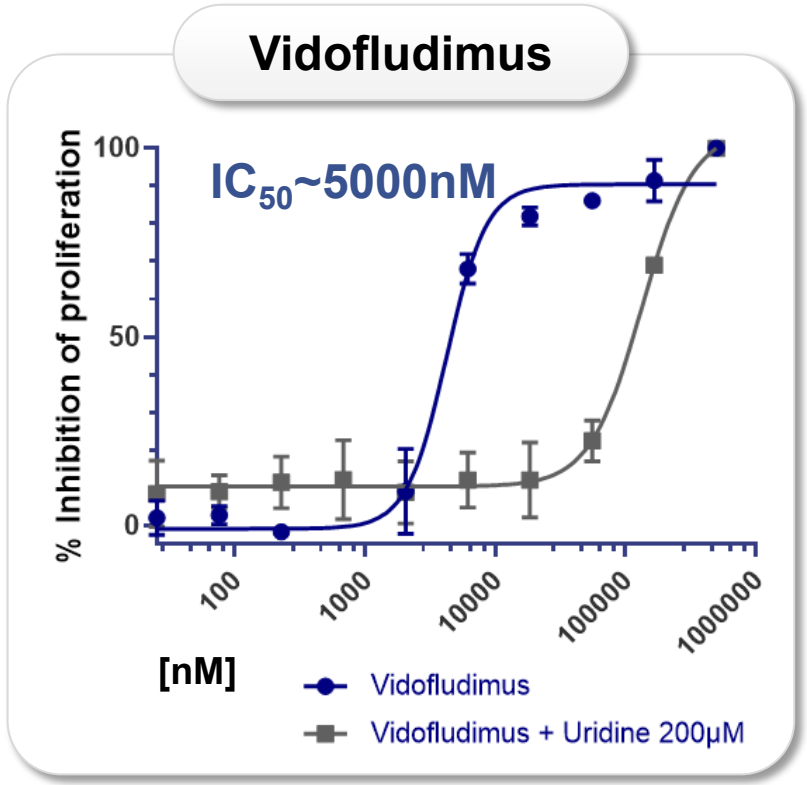
PTC844 DHODH Inhibitor Program

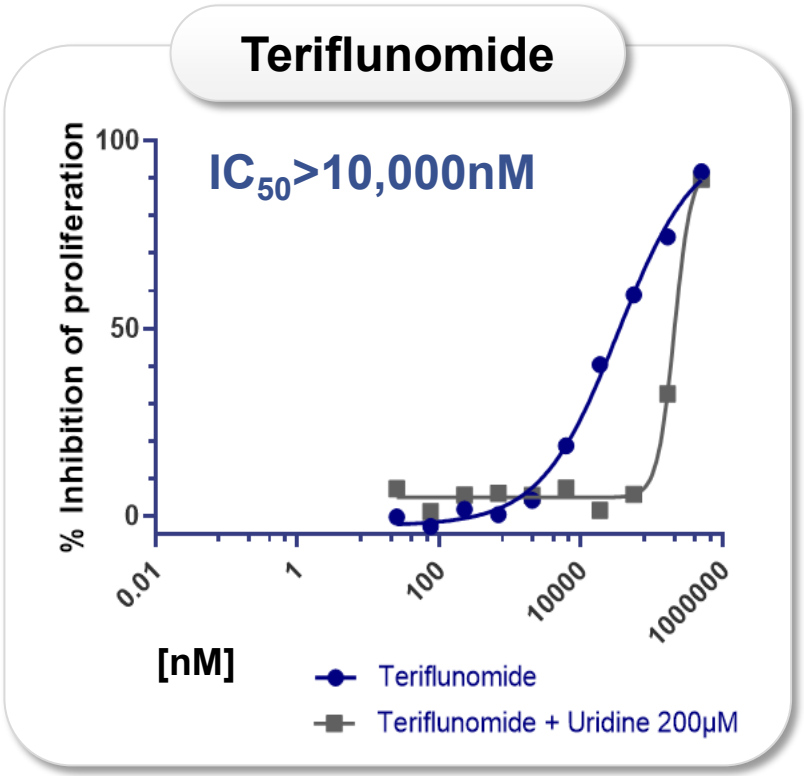
- DHODH plays a critical role in inflammation by regulating the proliferation of immune cells and modulating cytokine production
- Marketed DHODH inhibitors have limitations due to lack of specificity and associated toxicity
- PTC844 is a more potent and selective DHODH inhibitor, potentially improving efficacy, safety and tolerability
- PTC844 Phase 2a study initiated in Q3 2026
  - 12-week PK/PD study to assess treatment effect on biomarkers and inform target indications

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PTC844 Demonstrates Superior Potency and Selectivity Compared to Other DHODH Inhibitors







Phase 2a PK/PD study of PTC844 initiating Q3 2026

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Well Positioned for Success in 2026 & Beyond

Robust global commercial infrastructure

Lead product Sephience to support near- and mid-term growth

Innovative R&D platforms and pipeline

Strong financial position

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October 2026



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